RARE Patient Advocacy Summit
Presented by Global Genes
September 11-12, 2014
Conference Agenda

A rare diagnosis changes everything. It crashes plans and dreams, knocks you off your feet, and requires a continual investment of time and money as you try to determine what should be your next step. The purpose of the *RARE Patient Advocacy Summit* is to help patient ADVOCATES become successful ACTIVISTS and provide the discussion, insights, and tools to move down this advocacy path, equipped and prepared. The Summit will offer practical advice, case studies, and networking opportunities as we learn from one another.

Throughout the Summit you will:

- Hear directly from experts
- Better understand how to overcome the challenges facing caregivers
- Learn about new advances in science
- Learn ways to become an unstoppable advocate and an effective organization
- Become equipped and educated on successful lobbying
- Focus on drug development and how patients are playing a critical role
- Network, Network. Network!
- Connect with other advocates who share your passion for advocacy

By attending this two-day event, you will meet new friends and colleagues, gain a new perspective on the complexities and questions that need to be considered in order to become effective advocates for rare disease, and be prepared to help make advances in the rare disease space.

Thursday September 11, 2014
Day 1:

12:30 – Registration Opens

1:00 – Opening Remarks – *Wendy White, CEO, Siren Interactive*
1:15 – Expectations of Summit – *Nicole Boice, President, Global Genes*

1:30 – Session 1 – Caregivers: Strategies to Stay Afloat
Presented by Caregiver Action Network and Partners on the Path

Being a rare disease caregiver is all-important and all-consuming. To have the strength and stamina for your caregiving journey, you must take care of yourself. But we know how hard self-care can be! In this interactive session, John Schall and Jane Hamilton will examine the ocean of challenges that caregivers face, and strategies on how to stay afloat in those sometimes stormy seas! Most caregivers face many of the same challenges, so we can help each other by sharing common experiences. The session leaders will help you explore both how to build interior resilience that will help you cope, and how to link to outside resources that will help you solve caregiving challenges.

Session Moderators – John Schall, CEO, Caregiver Action Network (CAN)
Jane Meier Hamilton, MSN, RN, CEO and Founder, Partners on the Path

2:15 – Session 1 Deep Dives

- 1.1 – Asking for Help – *John Schall, CAN*
- 1.2 – Making Peace with What You Can't Control – *Jane Meier Hamilton, Partners on the Path*
- 1.3 – Challenges as a Family – *Rob Miller, Rob Miller Human Service Consulting*
- 1.4 – From Caregiver to Caregiver Advocate, Mobilizing Your Community – *C. Grace Whiting, National Alliance for Caregiving*
- 1.5 – Parent to Parent – *Lisa Raman, RN, Klippel-Feil Syndrome Alliance*
- 1.6 – Patient to Patient – *Cyndi Frank, National Gaucher Foundation*
- 1.7 – Maximizing Learning Potential for School Aged Children – *Tanya Johnson, Zenzaga*

3:00 – Break – *Sponsored by Pfizer*

3:10 – Innovations in Science Presentation Brief #1 – *RARE Science Founder & CEO, Christina Waters, PhD, MBA*

3:30 – Session 2 – The E-Patient Revolution
Presented by Health 2.0 and Smart Patients
It is common knowledge that the last two decades of the modern Internet have turned banking and retail commerce upside down. More quietly, the same period has seen isolated patients

with little access to health information transformed into networks of informed micro-experts who help each other get the best care possible. Their power stems from the fact that they now have access to massive amounts of information and can collaborate with each other in order to better understand it. Especially for the rare disease community, the processes of diagnosis, treatment, and even basic research are being improved directly by patients.

Session Moderator – Indu Subaiya, CEO and Co-Founder, Health 2.0

- Session Participant - Roni Zeiger, MD, Co-Founder, Smart Patients
- Session Participant - Julia Hallisy, Advocate, Founder and President, The Empowered Patient Coalition
- Session Participant - Gilles Frydman, Advocate and Co-Founder, Smart Patients

4:15 – Session 2 Deep Dives

2.1 – The Power of Putting Information into the Hands of the Patient – *Courtney Larned, CareSync*

2.2 – Patient Driven Research: Putting Patients at the Center of Clinical Trials, From Start to Finish – *Gilles Frydman, Smart Patients*

2.3 – Tools and Strategies to Fast-track Patient Engagement – *Julia Hallisy, The Empowered Patient Coalition*

2.4 – Benefits of Using Online Community Building Tools for Effective Advocacy and Support – *John Stamler, Ben's Friends*

2.5 - Move Beyond Anecdotes: A Blueprint for Patient-Driven Research – *Manu Kodiyan, Althea Health and Ronnie Sharpe, CysticLife*

5:00 – Innovations in Science Presentation Brief #2 – *American MedChem Nonprofit Corporation Founder, President and CEO, Robert Selliah, PhD*

5:20 – Session 3 – Shaping Regulatory Policies that Put Patients First

The FDA has sought to increase the patient voice in orphan drug-related regulation. One innovative patient group, Parent Project Muscular Dystrophy, recently submitted the first-ever draft guidance on drug development at the Agency's request. How did this come about? What was PPMD's role? What will the Agency do now? And what does this mean for your organization? Workshop participants will leave the session with practical tips for responding to new opportunities in shaping regulatory policy.

- Session Presenter – Pat Furlong, Founding President and CEO, Parent Project Muscular Dystrophy
- Session Presenter – Mark Krueger, MPH, President, Mark Krueger & Associates, Inc.

6:00 – Day One – It's a Wrap

Friday September 12, 2014
Day 2:

7:30 – Breakfast *sponsored by Walgreens*

8:00 – Opening Remarks – *Terry Cato, Senior Director, Disease State Product Management*
Recap Day 1 – *Wendy White, CEO, Siren Interactive*

8:15 – Session 4 – Patient-Centered Benefit-Risk Assessment, Why It Matters to You
Presented by FasterCures

Patients and caregivers facing life-altering conditions view the relative benefits and risks of medicines, devices, and other therapies differently than others in the medical ecosystem. In general, they are more willing to accept risks for even marginal benefits that extend life or improve quality of life. Until recently, patients' perspectives and preferences have received little attention from FDA regulators or industry sponsors – or even physicians. Thankfully, this is changing. A 2012 law that governs the Food and Drug Administration created the agency's first Patient-Focused Drug Development Initiative. The center in which medical devices are reviewed is also beginning to incorporate patient preferences into decision-making.

Learn from *FasterCures'* Director of Strategic Initiatives, Kim McCleary, about what these programs mean for rare disease advocates. You'll hear how patient organizations are mobilizing their communities to help inform decisions made by the FDA and industry sponsors so that treatments move more swiftly from the lab to the patients who need them. Tools created for this purpose will be shared, including lessons learned at the frontlines of two communities that have participated so far.

Session Moderator – Kimberly McCleary, Director of Strategic Initiatives, FasterCures
Session Participants

- Pat Furlong, Founding President and CEO, Parent Project Muscular Dystrophy
- Roy Zeighami, Founder and Board President, Sanfilippo Foundation for Children

9:00 – Session 4 Deep Dives

4.1 Mobilizing Your Community for Patient-Focused Drug Development - *Kimberly McCleary, FasterCures*
4.2 Natural History Translated into Policy - *Pat Furlong, Parent Project Muscular Dystrophy*
4.3 Risk v. Benefits: Tradeoffs for Treatments - *Roy Zeighami, Sanfilippo Foundation for Children*
4.4 Tools to Mobilize (Patient Information Tool) - *Eric Gascho, Assistant Vice President, Government Affairs, National Health Council*

10:00 – Break – *Sponsored by Shire*

10:10 – **Innovations in Science Presentation Brief #3 –** *Project Violet Founder, Jim Olson, PhD*

10:30 – Session 5 – The Unstoppable Charity

Have you ever wondered how to get more donors? Wished you had more successful fundraisers? Wanted your volunteers to become more involved? If you answered YES to any of these questions, you may want to check out this session. Based on the common principles that have helped network marketing companies and political campaigns grow, this session will outline how your charity can focus on engagement to further your mission.

Presented by - Keegan Johnson, CEO Zenzaga

In this interactive session, you will learn:

- The 10 parts of a solid charity foundation
- How to use "Effective Storytelling" to grow your charity
- The key to generating momentum
- A step-by-step approach to increasing member engagement
- How to avoid common mistakes that destroy member motivation
- Network marketing and political campaign secrets you can use today

11:15 – Session 5 – Deep Dives:

Strategy

5.1 - Mission, Goals and Strategic Planning – *Keegan Johnson, Zenzaga*
5.2 – Org to Org: Finding Common Ground within the Disease Community – *Nicole Boice, Global Genes & Lisa Schill, RASopathies Network USA*
5.3 – Where the Needs of Families and Healthcare Professionals Intersect – *Robert Miller, Rob Miller Human Service Consulting*
5.4 - Utilizing Social Media: Be an Effective #RareDisease Advocate in 15 Minutes – *Stephanie Fischer, Pharmaceutical Research and Manufacturers of America (PhRMA)*

Fundraising

5.5 – Creating a Comprehensive Development Plan: Resources and Revenue – *Susan Hoover Miller, CFRE, Susan Hoover & Associates*
5.6 – Grant Writing – *Leslie Perovich, CFRE, Pretend City*
5.7 – Venture Philanthropy: Is This an Option for my Disease Community? – *Debra Miller, CureDuchenne*

The Business of Being a Non-Profit

5.8 – Build a Board That Can Support Your Goals – *David LaGreca, VCG Governance Matters*
5.9 – Managing Your Organization's Multiple Relationships: Know When to Hold, to Be Bold, and When to Fold! – *Jean Campbell, JC Consulting*

The Concrete Stuff

5.10 - Legal: Best Practices – *Tim O'Connor, Esq. O'Connor Schmeltzer O'Connor*
5.11 - Finance/Accounting: Best Practices – *Deana L. Bowden, CPA, White Nelson Diehl Evans LLP*

5.12 – People Management: Employees, Volunteers, Building Leaders and Managing Conflict – *Andrea Epstein, Global Genes*

12:00 – Networking Lunch *Sponsored by Alexion Pharmaceuticals*

1:00 - Session 6 –
Transition and Transformation – Rare Disease in Adolescence and Adulthood

Being an adolescent or adult with a rare disease can be a time of many changes - changes in health status, healthcare systems, education, work, living arrangements, family and relationships. Some of these changes - transitions - can be planned and organized, while other changes - transformations - may have more to do with who you are, how you make decisions about your health, and how you (and your family) live with your rare condition. This session will provide insights, ideas and resources to help you and your family negotiate this exciting and challenging time.

Presented by Dr. Maya Doyle, Children's Hospital at Montefiore

1:45 – Session 7 – Preparing for Success; Lobbying at the State and Federal level

It is never too early to start preparing for World Rare Disease Day. For advocates who plan to lobby their Members of Congress or state legislators in either their district offices or at their capitol offices, this session on best practices will be extremely valuable. Time with Members and/or staff is usually quite limited, and knowing how to effectively prepare so that you are able to get the most out of your meeting is vital to your success. Learn how to prepare concise meeting materials, communicate appropriately with legislative staff, organize your meeting participants for maximum efficiency and hear real world experiences and advice from those who have frequently participated in these kinds of meetings.

Session Participants:

- Jennifer Bernstein, Horizon Government Affairs
- Julia Jenkins, Rare Disease Legislative Advocates
- James Romano, Patient Services, Inc.

2:30 – Break – *Sponsored by Shire*

2:40 – **Innovations in Science Presentation Brief #4 –** *Immusoft Founder and CEO, Matthew Scholz, PhD*

3:00 – Session 8 – Putting into Practice – 21st Century Cures

The House Energy & Commerce Committee is currently working on a 21st Century Cures Initiative. The Initiative is a bipartisan effort to take a comprehensive look at what steps Congress can take to accelerate the pace of cures in America. The Committee is examining the full arc of this process – from the discovery of clues in basic science, to streamlining the drug and device development process, to unleashing the power of digital medicine and social media at the treatment delivery phase. The Committee is gathering information on how to close gaps

between advances in scientific knowledge about cures and the regulatory policies created to save more lives.

We will talk in more detail about the initiative, what it means for you, and then provide you with an opportunity to weigh in on this issue with the Committee.

Presented by: Jennifer Bernstein, Horizon Government Affairs

3:30 – Session 9 – The Must-Have Collaborations for Successful Drug Development

In rare disease communities, getting drugs to market as quickly as possible is a team effort that requires skillful collaboration between four groups of stakeholders: the biopharmaceutical industry, academia, government and patient groups. In this hands-on workshop, representatives from each of these groups will talk about what they bring to the table, what makes them successful and what their stress points are. After their presentations, groups will have twenty minutes to develop a 'quick pitch' in a role-playing exercise that allows participants to take on the role of one of these stakeholders. This is a great way to hone your collaboration skills and develop a greater understanding of the various points of view from the key players in the drug development process.

A white paper will be developed as an output from this session.

Session Moderators **–** Barbara Wuebbels, Vice President of Patient Advocacy and Medical Affairs, Audentes Therapeutics and Wendy White, Founder and CEO, Siren Interactive

Session Participants:

- Dr. Steve Groft, former Director, NIH Office of Rare Diseases Research
- Hudson Freeze PhD, Professor and Director, Human Genetics Program
  Sanford Children's Health Research Center, Sanford-Burnham Medical Research Institute
- Matt Wilsey, President, Grace Wilsey Foundation
- Jonathan Jacoby, Hide & Seek Foundation, SOAR-NPC
- Katherine Rauen, MD, PhD, University of California Davis MIND Institute

5:00 – **Day Two – It's a Wrap**

5:00 – **Margaritaville Networking Reception** – Lighthouse Courtyard
Proudly Sponsored by Cincinnati Children's Hospital
In Partnership with Mauli Ola Foundation